[Billing Code 4140-01-P]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Prospective Grant of Exclusive License: Therapeutics for the Treatment of Lysosomal Storage Disorders

AGENCY: National Institutes of Health, HHS.

ACTION: Notice.

SUMMARY: This is notice, in accordance with 35 U.S.C. 209 and 37 CFR 404, that the National Institutes of Health (NIH), Department of Health and Human Services (HHS), is contemplating the grant of an exclusive license to practice the following invention as embodied in the following patent applications:

1. U.S. Provisional Patent Application No. 61/365,712, filed July 19, 2010
   HHS Ref. No. E-294-2009/0-US-01
   Titled: Use of Delta-Tocopherol for the Treatment of Lysosomal Storage Disorders
2. PCT Application No. PCT/US2011/044590, filed July 19, 2011
   HHS Ref. No. E-294-2009/0-PCT-02
   Titled: Use of Delta-Tocopherol for the Treatment of Lysosomal Storage Disorders

3. European Patent Application No. 11741023.3, filed July 19, 2011

   HHS Ref. No. E-294-2009/0-EP-03

   Titled: Use of Delta-Tocopherol for the Treatment of Lysosomal Storage Disorders

4. U.S. Patent Application No. 13/810,774, filed January 17, 2013

   HHS Ref. No. E-294-2009/0-US-04

   Titled: Use of Delta-Tocopherol for the Treatment of Lysosomal Storage Disorders

5. U.S. Provisional Patent Application No. 61/679,668, filed on August 3, 2012

   HHS Ref. No. E-050-2012/0-US-01

   Titled: Cyclodextrin for the Treatment of Lysosomal Storage Diseases

6. PCT Patent Application No. PCT/US2013/053527, filed on August 3, 2013

   HHS Ref. No. E-050-2012/0-PCT-02

   Titled: Cyclodextrin for the Treatment of Lysosomal Storage Diseases

7. U.S. Provisional Patent Application No. 61/727,296, filed November 16, 2012

   HHS Ref. No. E-148-2012/0-US-01

   Titled: Tocopherol and Tocopheryl Quinone Derivatives as Correctors of Lysosomal Storage Disorders

8. PCT Application No. PCT/US2013/070156, November 14, 2013

   HHS Ref. No. E-148-2012/0-PCT-02

   Titled: Tocopherol and Tocopheryl Quinone Derivatives as Correctors of Lysosomal Storage Disorders,

to Vtesse, Inc., having a place of business in Cambridge, Massachusetts, United States of America. The patent rights in these inventions have been assigned to the United States of America.

DATES: Only written comments and/or application for a license which are received by the NIH Office of Technology Transfer on or before [INSERT DATE FIFTEEN (15) DAYS FROM DATE OF PUBLICATION OF NOTICE IN THE FEDERAL REGISTER] will be considered.

ADDRESSES: Requests for a copy of the patent application, inquiries, comments and other materials relating to the contemplated license should be directed to: Suryanarayana Vepa, Office of Technology Transfer, National Institutes of Health, 6011 Executive Boulevard, Suite 325, Rockville, MD 20852-3804; Email: vepas@mail.nih.gov; Telephone: (301) 435-5020; Facsimile: (301) 402-0220.

SUPPLEMENTARY INFORMATION: These technologies relate to the use of cyclodextrin (CD), delta-tocopherol and their derivatives for the treatment of lysosomal storage disorders (LSDs). LSDs are inherited metabolic disorders caused by a deficiency in lysosomal enzymes, of which approximately fifty (50) have been described to date. These diseases usually affect children, many of whom die within several years of birth and some following years of dealing with symptoms of the disease that may include developmental delay, movement disorders, seizures, dementia, deafness and blindness. LSDs affect a significant number of individuals and some can be treated with enzyme-

replacement therapies. However, because enzymes cannot cross the blood–brain barrier, replacement therapeutics are unable to address the central nervous system manifestations of the disorders. The inventors have identified an unexpected and previously unrecognized use for delta-tocopherol, which is a form of vitamin E, in the treatment of diseases and conditions related to LSDs. Further, the inventors showed that CD (alpha-, beta- and gamma-CDs) in combination with delta-tocopherol synergistically/additively reduced cholesterol accumulation in cells derived from patients suffering from Niemann Pick Type C disease (NPC) and Wolman diseases. The inventors have also discovered that tocopherol and tocopheryl quinone derivatives with side chain modifications (such as terminal tri-halogenated methyl groups) exhibit improved pharmacokinetics, modulation of mitochondrial potential and restoration of some LSDs phenotypes. These technologies can be used to develop novel therapeutics for LSDs including NPC, Wolman, Niemann Pick Type A, Farber, TaySachs, MSIIIB and CLN2 (Batten) diseases.

The prospective exclusive license will be royalty bearing and will comply with the terms and conditions of 35 U.S.C. 209 and 37 CFR 404. The prospective exclusive license may be granted unless, within fifteen (15) days from the date of this published Notice, NIH receives written evidence and argument that establishes that the grant of the license would not be consistent with the requirements of 35 U.S.C. 209 and 37 CFR 404.

The fields of use may be limited to “Use of cyclodextrin, delta-tocopherol, or derivatives thereof, alone or in combination, for the treatment of lysosomal storage disorders in humans.”

Properly filed competing applications for a license filed in response to this notice will be treated as objections to the contemplated license. Comments and objections submitted in response to this notice will not be made available for public inspection, and, to the extent permitted by law, will not be released under the Freedom of Information Act, 5 U.S.C. 552.

Dated: September 12, 2014.

_____

Richard U. Rodriguez,
Director,
Division of Technology Development and Transfer,
Office of Technology Transfer,
National Institutes of Health.

[FR Doc. 2014-22210 Filed 09/17/2014 at 8:45 am; Publication Date: 09/18/2014]